Transcript: Ofev Roundtable Event, April 22, 2025, Medicare Drug Price Negotiation Program Public Engagement Events

This transcript was lightly edited for readability.

Introductory Remarks

Moderator, RTI International

And thank you to the participants for coming today. I'm **[MODERATOR]**, and I'm from RTI International, and I also want to introduce my colleague, **[SECONDARY MODERATOR]**, who you may hear from at a few points in the discussion today.

The Centers for Medicare & Medicaid Services, CMS, is convening this patient-focused, roundtable event, and others as part of the Medicare Drug Price Negotiation Program.

The purpose of today's event is to hear from you all, a group that may include patients, caregivers, and patient advocates, about your experiences with the conditions and diseases treated by Ofev, with Ofev itself, and with other medications for the same conditions.

If you wish to share input on other topics related to the Drug Negotiation Program, please do so through the mailbox at IRARebateAndNegotiation@cms.hhs.gov, and we'll be showing that address again at the end, so that you have it handy.

Okay. The information that's shared during these events will help CMS understand patients' experiences with the conditions and diseases treated by the selected drugs, patients' experiences with the selected drugs themselves, and patients' experiences with other drugs that are used to treat the same conditions as the selected drug. CMS may use this information in negotiating Medicare pricing with the manufacturers of selected drugs, and your experience and perspectives are very important to us. We genuinely appreciate your time today.

Let's go ahead and watch a brief welcome video from CMS leadership so that you can hear directly from them about how much they value your time and input.

CMS Remarks

00:01:56

Steph Carlton, Deputy Administrator and Chief of Staff, Centers for Medicare & Medicaid Services

Greetings, everyone. I'm Steph Carlton, the Deputy Administrator and Chief of Staff at the Centers for Medicare & Medicaid Services, or CMS. CMS administers Medicare, our country's federal insurance program, for more than 65 million older Americans and people with disabilities.

I deeply appreciate each one of you for taking the time to join us today. Lowering the cost of prescription drugs for Americans is a top priority of President Trump and his administration. As the second cycle of negotiations begins under the Trump administration, CMS is committed to engaging with stakeholders for ideas to improve the Negotiation Program.

In January 2025, CMS announced the 15 Medicare Part D drugs selected for the second cycle of price negotiations. Medicare's ability to negotiate directly with drug companies will improve access to some of the costliest drugs while fostering market competition and continuing innovation.

Our priority in negotiating with participating drug companies is to come to an agreement on a fair price for Medicare. Promoting transparency and engagement continues to be at the core of how we are implementing the Medicare Drug Price Negotiation Program. And that is why the process for negotiation engages you, the public.

This event is part of our effort to hear directly from a range of stakeholders and receive input that's relevant to the drugs selected for the second cycle of negotiations. Thank you again for joining us. Your input matters. And next, stay tuned to hear from the event moderator to give you more details on what to expect during this event.

00:03:52

Moderator, RTI International

Great. I also want to make you aware that staff from CMS will be sitting in on this event so that they can hear your experiences and opinions directly from you. Let me hand it over to them for a moment, so that they can say hello. **[CMS STAFF]**?

00:04:08

CMS Staff

Thanks, **[MODERATOR]**, good morning and welcome. We have staff here today from the Medicare Drug Price Negotiation team, and we want to thank you very much for participating today. We are looking forward to the discussion. We are going to go off camera so that you all can focus on the discussion. But we are going to be here and listening.

Housekeeping

00:04:30

Moderator, RTI International

Great. Thanks, **[CMS STAFF]**.

Okay, well, before we get started, I just want to review some housekeeping items and ground rules so that everyone knows what to expect.

First off, participation. We hope that you will contribute your perspectives throughout the session. However, if questions arise that you don't want to answer, that's totally okay.

In terms of background, please just minimize any background noise by silencing your cell phone and any other devices you have in the area. Also, please just mute yourself when you're not speaking.

In terms of privacy, the discussion is not open to the press or the public. We'll be using first names only during the discussion to protect your privacy. Please don't share any unnecessary personally identifying information or personal health information during the discussion.

We are audio and video recording today. But these recordings will not be shared publicly. Following the event, CMS will prepare transcripts that have participant names and identifying information removed, and these will be available to the public.

I also want to highlight a few things to keep in mind for our discussion. Video, thanks in advance for keeping your video on throughout the discussion. I really appreciate that. It helps me see when folks are ready to speak and also to see some nonverbal cues.

And in terms of timing, this session will last about one hour and 30 minutes. I have a discussion guide in front of me to help me keep on track. I have a lot of different topics that I want to cover with you all, so I may need to redirect our conversation, or cut a particular conversation short at times. It's not because I wouldn't love to hear more about that topic. It's just so that I can make sure that I cover everything.

In terms of technical assistance. If you get disconnected, please just attempt to rejoin. If you can't connect, please reach out to IRADAPStechsupport@telligen.com, and that's the address that **[TECH STAFF]** just put into the chat for you to have handy.

If you do need to step away briefly during our discussion, that's totally okay. Just turn your camera and your microphone off and rejoin when you're able to. You don't need to tell me that you'll be away from your computer. Please just return to the discussion when you're able to.

Please try to speak one at a time. I may occasionally interrupt when two or more people are talking and that's just to make sure that everyone gets a chance to talk, and that everyone's comments are accurately recorded.

Please feel free to use the raise hand feature in Zoom. I know that you've already found that feature during our tech checks. With only two participants, hopefully we won't have any trouble making sure that everybody gets to chat about each topic. And this will help us know if you do raise your hand when you'd like to contribute to the discussion. And you can always also add comments into the chat if you'd like.

Lastly, your comments and experiences will differ. We want to know what each of you honestly thinks about the topics we discuss.

Does anyone have any questions before we get started about those housekeeping items?

Okay, all right. Well, hearing none, let's jump right in. Before we do that, I want to give you a heads up that I am a bit under the weather, so please bear with me. You may be able to tell from my voice, but we'll get through it.

Okay, so I would like to begin our discussion by asking you to introduce yourself. Can you please just tell me briefly, in about 30 seconds, your first name, the condition or conditions that Ofev treats that you have experience with, and whether you'll be sharing personal experiences or those of a loved one, or whether you're sharing patient experiences from the perspective of a patient advocate.

Maybe we'll start with you, **[Participant 1]**.

Discussion

00:08:51

Participant 1 (registered as a representative of a patient advocacy organization)

Thank you. My name is **[Participant 1]**. I am here on behalf of a patient advocacy organization that advocates on behalf of health issues affecting largely older Americans.

00:09:04

Moderator, RTI International

Great, thanks for sharing that. What about you, **[Participant 2]**?

00:09:08

Participant 2 (registered as other)

I'm here on behalf of the American Thoracic Society. I'm a pulmonologist who takes care of patients with advanced lung disease, and in particular patients with various forms of pulmonary fibrosis. I am from the provider side, here as an advocate.

00:09:26

Moderator, RTI International

Right. Okay, thanks. Now that you all have introduced yourselves briefly, I would like for you to use the chat feature to share one piece of information that will be helpful for me to know going forward. Have you or any of your loved ones taken Ofev whether currently or in the past? And if you just please enter a yes or no in chat, I would appreciate it.

Okay.

All right, **[Participant 2]**, that's helpful to know.

Okay, thanks. So, thanks again for introducing yourselves and telling me what experiences you'll be drawing from today. Let's start by talking about patients' experiences with the conditions treated by Ofev.

We know that there will be a lot to talk about with this question, so please feel free to just raise your hand when you're ready to chat, and again, you can always enter comments in the chat as well.

I do want to give you a heads up that for each topic, I'll first be asking about idiopathic pulmonary fibrosis. Then I'll be repeating my question for progressive pulmonary fibrosis, and then I'll repeat the question yet again, so that we can cover systemic sclerosis-associated interstitial lung disease.

Given that these condition names are a bit of a mouthful, I may sometimes just refer to the conditions by their acronyms. IPF for idiopathic pulmonary fibrosis, PPF for progressive pulmonary fibrosis and SSc-ILD for systemic sclerosis-associated interstitial lung disease.

Okay, in general, how does idiopathic pulmonary fibrosis affect patients' day-to-day lives? **[Participant 2]**?

00:11:26

Participant 2 (registered as other)

Sure. So idiopathic pulmonary fibrosis is a condition where there's scarring that develops in the lung tissue. So, when we think about our lungs, we've got sacs, and these sacs hold air, and those sacs develop scarring in them. And so, you get scarring that develops in the air sacs, and that makes it challenging for patients on a number of reasons. One is that the lining between the air sacs where the air comes in and the blood vessels is thickened and scarred, and so that makes the exchange between the gas from the blood vessels to the air sacs much more difficult.

So, patients have difficulty with their oxygen levels going down low, and they can also develop difficulties exchanging their carbon dioxide. So, they will have difficulty when they exercise,

difficulty with shortness of breath. Oftentimes they can get short of breath at rest. They might need oxygen itself to help supplement the limitation in the exchange of oxygen between those air sacs called alveoli and the bloodstream, and then, when it gets quite advanced, they have difficulty with their carbon dioxide, because it actually shrinks the size of the lung, because you imagine that all the air sacs shrink down, and so, as the lung becomes more scarred down, it makes it more challenging for patients to actually blow off the carbon dioxide that they've developed.

So, these patients can get very limited. They are short of breath doing any of their activities of daily living. Some of them become fully dependent on other people for care. They might be entrapped in their home because of the current way that oxygen supplementation is under the competitive bidding process because there's no access to liquid oxygen in the United States anymore. So, these patients will be at risk of death. And then they need lung transplants, sort of the only option that we have to fix the problem.

00:13:29

Moderator, RTI International

Thank you, **[Participant 2]**. What about you, **[Participant 1]**? Can you tell me a little bit about how IPF affects patients' day-to-day lives from your perspective?

00:13:39

Participant 1 (registered as a representative of a patient advocacy organization)

Yeah, I think that was a pretty thorough, comprehensive description. I don't know that I have anything to add to that other than to say that, yes, these folks do not get better, and the medication really only helps to slow the process, inevitable process of decline.

But obviously it's extremely valuable for quality of life, and hopefully, if they can, they are eligible for a transplant to buy them the time that they need to get to that point.

00:14:14

Moderator, RTI International

All right. And now **[Participant 1]**, we'll turn back to you first then, for progressive pulmonary fibrosis, if you have any insights to share regarding daily living experiences for that condition.

00:14:26

Participant 1 (registered as a representative of a patient advocacy organization)

Not really. No, thanks.

00:14:29

Moderator, RTI International

Got it. What about you, **[Participant 2]**? Do you have anything that you want to share regarding PPF?

00:14:35

Participant 2 (registered as other)

No, it's similar. The disease process is different. What's driving it is different. But the end stage manifestations of it are basically the same.

00:14:46

Moderator, RTI International

Okay, that's helpful to know. And then let me ask again, for systemic sclerosis-associated interstitial lung disease. Any differences in how that affects daily living activities?

00:15:00

Participant 2 (registered as other)

Well, some patients with scleroderma-associated ILD will have more complications related to their scleroderma itself. So, it's basically where the interstitial lung disease or the scarring of the lungs is caused by autoimmune disease, so scleroderma.

The problem with scleroderma is that scleroderma has multiple systemic effects. So, it's not just in the lung. It is in the lung, and it's in more than one compartment of the lung. But it has other areas that it's affecting, too. So, these patients might have skin thickening issues. They might have problems with their hand swelling. They might have problems with their esophagus. They might have problems with their gastrointestinal tract. So, there's more problems that they're dealing with.

So essentially, the medication was approved for patients with idiopathic pulmonary fibrosis. And then there's two subsequent trials looking at progressive pulmonary fibrosis. Similar. But not caused by idiopathic pulmonary fibrosis, and then scleroderma-associated, and so that's sort of why the label has been expanded. But the end stage sort of manifestations of the disease are fairly similar across the board.

00:16:11

Moderator, RTI International

Great. Thank you for that information.

So back to IPF, what aspects of idiopathic pulmonary fibrosis are most important to patients to have managed or treated?

[Participant 1], let me turn to you first on this one?

00:16:30

Participant 1 (registered as a representative of a patient advocacy organization)

Yeah, I think again, it is just to slow down the terrible progress of this illness. It is really to ensure that they can buy the time that they need. And there are other treatments, but this particular medication, the studies I've seen indicate that it's one of the more effective ones. Access is critical. Just again, for quality of life, the ability to breathe, we take for granted. And people are struggling with this, and every breath is a chore. So, it is really important to maintain whatever quality of life they can for as long as they can, maybe in hopes that a stronger treatment will come along before it's too late, or that they will actually have the option to have some surgery and address it that way.

00:17:40

Moderator, RTI International

Thanks, **[Participant 1]**. **[Participant 2]**, what aspects of IPF are most important to patients to have managed or treated?

00:17:48

Participant 2 (registered as other)

I mean, it's a multitude of things. I think one is, you get the diagnosis and a lot of people view it as they've gotten a death sentence, because essentially, this is a progressive disease that does not get better.

The problem with scarring is once you develop scarring, scarring doesn't go away. So, I always tell patients, this is a place I cut myself when I was in 5th grade. I still have this scar here. That's what's happening in your lungs. So, the scar happens in the lungs. The scar doesn't go away, so, preventing the scar from developing and preventing the scar from progressing is the only thing that we have, at this point in time, because we can't make scars go away.

In terms of the day-to-day things that the patients are mostly dealing with, again, it's limitations. As **[Participant 1]** eloquently put, every breath is a struggle. Every breath is a struggle. These patients are breathing so hard that they lose weight because they've developed pulmonary cachexia. So, they basically will burn through so many calories just breathing at rest that they lose weight because of it. They get debilitated. They get weak because they cannot do their activity [inaudible]. They can't do things, and it's not only... I would say I'm a transplant pulmonologist. So, I would take care of patients before transplant, and we're transplanting 30- and 40-year-olds with pulmonary fibrosis. Familial fibrosis is a genetic form of... this is very similar to idiopathic pulmonary fibrosis. It sort of is an idiopathic pulmonary fibrosis, but it's through a genetic mechanism, and we're transplanting like 30- and 40-year-olds with this. So, you have a 34-year-old that can't take their kid to school anymore because they have this. They can't go to work anymore because they have this. So, these are big impacts. I think quality of life, giving patients the ability to breathe, preventing them from getting worse, I think those are big things. And these medications, they don't take the scarring away, but they can extend the time that patients are not having more advanced scarring, which can improve quality of life for a long period of time.

00:20:02

Moderator, RTI International

Thank you, **[Participant 2]**. And what about progressive pulmonary fibrosis? What aspects of PPF are most important to patients to have managed or treated? Or, again, is it pretty similar?

00:20:15

Participant 2 (registered as other)

Yeah, the same. I think that PPF is probably a more broad definition in terms of the disease ranges. So, you're going to have more impact on Americans throughout the entire spectrum. I think idiopathic. There's familial IPF, and those patients can be younger. But mostly IPF is in the older age category. But PPF can be in a much younger category. And patients with IPF are oftentimes still working at the time they get their diagnosis. Patients with IPF are wanting or some of them have just gotten into retirement, and they want to enjoy their life, and now they can't anymore. They've worked their entire lives, and now they can't because they get this horrible devastating disease.

PPF, we tend to see a little bit younger, so these patients oftentimes will have young families that they're taking care of, and so that will impact them. It'll impact their children. They're worried about dying. They're worried about not being around for their children. They worry about not being able to work. They don't want to go on disability, but now they have to because they've developed pulmonary fibrosis. These are big issues that these patients are dealing with.

00:21:37

Participant 1 (registered as a representative of a patient advocacy organization)

Yeah, I would just add that, that was a good point there about the impact on families. Depending on the progression of the illness, these folks may require substantial in-home support and services, just to remain at home, and whether it's a burden on the family or a burden on the pocketbook, the more that you can slow the progression of the disease, the more you can forestall some of those challenges, but it is certainly definitely an impact, not just on the patient, but on all those who care for them.

00:22:19

Moderator, RTI International

Thanks for that addition, **[Participant 1]**.

And what about SSc-ILD? What are the aspects of that condition that are most important to patients to have managed or treated?

[Participant 2], I think you might be the one representing.

00:22:38

Participant 2 (registered as other)

It'd be very similar. The ILD components are going to be similar regardless. It's just that these patients have even more problems. Again, this is a young population as well, oftentimes that has this. Not always, but it is a young population that has it.

All these diseases can also develop pulmonary vascular disease, which is high blood pressure in the lungs as well, because when the scarring gets advanced, they can develop high pressure in the lungs, and so, as the scarring gets worse, they can go into heart failure, as well, and so preventing the scarring from getting worse can help prevent some of the heart failure. All these diseases, I think the other one that we haven't touched on too much is the oxygen requirement. If you can prevent the scarring from getting worse, you need less oxygen. So, the problem with oxygen in the United States right now is that we don't have access to high flow liquid oxygen in the outpatient setting anymore because of the competitive bidding process for oxygen. Oxygen is not a durable medical equipment, but it's in the DME category. And so there's been a race to the bottom, and we have less quality options for oxygen delivery for my patients, so I cannot prescribe my patients liquid oxygen, so they have either tanks which are very heavy and hard to carry around, regardless of the form of pulmonary fibrosis, and they are homebound because the DME companies basically get a capitated payment, and they bring not sufficient tanks for them to be able to go out and do their activities of daily living. So, if the scarring gets bad to the point that they need high flow oxygen, which is all of these patients eventually, now they're basically imprisoned in their home. And that is hard for patients, regardless of whether it's scleroderma, progressive pulmonary fibrosis, IPF, whatever the etiology is, that's really hard for patients. If we can get these medications more affordable for patients, we can get these medications, more accessible for patients, not going to prevent that problem because it's not fixing the oxygen marketplace. But it might prevent the number of patients that actually need these higher flow amount of oxygens, so that they can actually live their life a little bit better.

00:24:47

Moderator, RTI International

Thanks, **[Participant 2]**.

Okay, I appreciate all the input you've already shared. Now, I want to turn our attention to talking about experiences with Ofev.

In addition to Ofev, I want to note that I'm also interested in knowing about patients' experiences with other medications that are like Ofev for each of the health conditions that we're talking about here.

We sometimes, as you may know, refer to these other drugs used to treat the same condition or symptoms as therapeutic alternatives. So, you may hear me use that language.

So, when patients are considering potential medications for IPF, what matters to patients the most?

[Participant 1], do you want to start with that one?

00:25:38

Participant 1 (registered as a representative of a patient advocacy organization)

Well, it's pretty basic, I think, it's the cost, access, and ease of use and efficacy. I understand there's basically two modes of treatment. There are drugs similar to Ofev. And then there are sort of steroidal treatments. I think, from the patient perspective is, can I afford it? Is it easy to use? And is it available to me? And is it going to be the best outcome in terms of slowing the progress of the illness?

I do believe that, from what I've seen, and I defer to **[Participant 2]** in this, that this is not my deep expertise, but that Ofev has checked a lot of those boxes for folks that are struggling with these illnesses.

00:26:50

Moderator, RTI International

Okay, let me turn to you now, **[Participant 2]**, when considering potential medications for IPF, what matters to patients the most?

00:26:58

Participant 2 (registered as other)

I think the thing that matters to patients is more, what is the impact on the progression of the disease going to be? And then how well they tolerate it, for IPF. So IPF has a therapeutic alternative, Esbriet or pirfenidone is the therapeutic alternative for IPF. They're fairly similar, in terms of their response, we think. They have somewhat similar side effect profiles. Phototoxicity is higher with the pirfenidone. Esbriet has higher risk of cardiac toxicity. So, you might pick pirfenidone if somebody has some underlying cardiovascular disease, like coronary artery disease, but both of them can cause some gastrointestinal discomfort.

When choosing to put somebody on this medicine, it's more of what is their disease progression potentially going to look like, and it is a bit of a leap of faith. We're going to put you on a medicine that you're not going to feel better with, like the medicine doesn't actually make patients feel better. So interestingly, sort of different compared to some other diseases in some other states where we actually put people on a medicine, and the hope is that the medicine is going to make them feel

better. These medicines don't actually make you feel better. They probably actually make you feel worse. But you take it because it's preventing the fibrosis. That's the only thing we have to prevent the fibrosis.

With idiopathic pulmonary fibrosis, those are the two treatment options. And there's currently nothing else on the market. We're expecting results from PDE4B inhibitor, probably around May of this year. They have a positive top line result, and so there might be another therapeutic alternative there, but it's been studied on top of Ofev or pirfenidone background therapy. And so, it's not going to be a replacement for it. And so, it would potentially be an addition to it. So, in the baseline category for that drug, there was, I think, like 80% of patients that were on background therapy with an antibiotic and either pirfenidone or Ofev.

So, I don't think you have a whole lot in terms of therapeutic alternative, aside from pirfenidone. And it's mostly side effects that we're deciding on when to use it. For progressive pulmonary fibrosis and for scleroderma, just because I know you're going to ask about those too, diseases are so similar. So for progressive pulmonary fibrosis, pirfenidone is not approved. For scleroderma, pirfenidone is not approved. So, there's no therapeutic alternative for those two.

There are other medicines that we can sometimes use depending on the type of pulmonary fibrosis. So, if it's related to exposure to bird feathers, for example, or something like mold, you could use immunosuppressants. If it's related to scleroderma, you could try using immunosuppressants. But still, the whole idea with the progressive pulmonary fibrosis disease category is that this was studied in addition to all those medicines. So, if patients are on immunosuppressants for an immune responsive pulmonary fibrosis, these medicines still work to slow progression. So, it doesn't matter what the cause of the fibrosis is, these medicines work to slow progression. And so, they don't have another therapeutic alternative. You're going to want to put your patient on this. And the therapeutic alternative is basically, you're going to get worse. And you're going to die.

00:30:45

Moderator, RTI International

Thanks. You definitely anticipated some of my different questions, even beyond just talking about the other two conditions, because I did want to get into what the different therapeutic alternatives that patients might be taking for these different conditions.

So, back to the original question of what matters most to patients when they're considering potential medications for these conditions, heard some different things, and in some cases, it doesn't sound like there's a ton of choices. But I did want to ask about some other potential things that might matter to patients a lot like, for example, how quickly it helps, how frequently it needs to be taken. It's convenience. It's ease of use. Are any of those relevant factors?

00:31:36

Participant 1 (registered as a representative of a patient advocacy organization)

Oh, yeah, I think those are definitely relevant factors. And I think it's ease of use is always great, particularly amongst older populations, where they may be on multiple medications. I mean, it's great that it's a pill form, and that it's pretty easy to comply with. Compliance is important with any of these types of medications. But I guess I would also say, I mean people who receive this diagnosis, and then they say, well, we have this medicine. It's not going to make you better, but it's going to keep you from getting worse more slowly. And then, you know, you're confronted with the other factors and access to it. And then, it is a quandary for a lot of patients that do I really want to

go through with this? And so, there's an emotional element to this as well. I think, obviously, those are decisions between the patient and their doctor. But I think it's very important that access to this medication, for all the reasons we've discussed, is there.

00:32:58

Moderator, RTI International

Anything else to add about what's important to patients when considering these medications?

00:33:02

Participant 2 (registered as other)

Yeah, I think that the two ones, in particular, sort of Ofev is BID, so twice a day dosing, and pirfenidone is three times a day dosing. So Ofev has a little bit easier use on patients, just because three times a day is a little bit challenging for some people. You take your pill with you to lunch, but then you get busy at work, and then you forget what's going on. You don't take your pills, so Ofev is a little bit easier, because it's a morning and night medicine. So, people have a little bit better compliance with the twice a day versus three times a day medicine all the time.

Again, both of them have gastrointestinal discomfort as a side effect, and so I think that that ends up being probably the hardest thing in terms of decision, because if patients are developing weight loss and poor appetite because of the pulmonary fibrosis... So, one of the things that happens is when the pulmonary fibrosis can get very advanced, patients don't have a lot of functioning lung. When you eat a meal, your stomach gets bigger. That increase in the size of the stomach with a normal meal is so much that patients will feel more short of breath because they ate.

They don't want to eat, because the eating makes them more short of breath. Because the fibrosis is so advanced. So then it's hard to put them on a medicine that's going to make them lose their appetite because they're going to become even more malnourished, problematic that way. So sometimes we will not use it because patients are so advanced that we worry about that side effect.

Sometimes, if they're going to transplant, and they're sort of already there, we will worry about not using it because we're just going to take them to transplant soon anyways. I think the other one which we haven't touched upon is, patients have access, and then they lose access. What happens to those patients? There's not a whole lot of data out there, but anecdotally, as a transplant pulmonologist, I will tell you that I see a lot of patients who've been on these medicines, and then they lose access to these medicines, and then they have an exacerbation.

So, the way the exacerbation of pulmonary fibrosis works is that the fibrosis just gets worse all at once. Patients are okay. They're stable. They're on two liters of oxygen, or they're not on oxygen. And then the fibrosis just gets worse. Now they're in the hospital. Now they're on 20 liters of oxygen. Now we're talking about death. We're talking about, can we rapidly work them up for transplant? We're hoping they get better. We don't know if they're going to get better. They're probably not going to get better. So, losing access to the medicine can, at least, I believe, anecdotally, and a lot of our transplant pulmonologists will tell you the same thing anecdotally, cause people to exacerbate, and exacerbations associated with morbidity, associated with mortality, increased need of a transplant. Progressive fibrosis, you get an exacerbation, even if you get better, your lung function is now worse. Even if you get better. It's a stepwise function. You start up here. You have an exacerbation. You're now here.

Best case scenario, you're here. You're not back up here. That means you're on more oxygen. You're less functional. You're more debilitated. You're not able to interact with your family much. You're not able to go to work anymore. So, losing access to these medicines is a really important thing for these patients, as well.

00:36:27

Participant 1 (registered as a representative of a patient advocacy organization)

If I could piggyback on that issue just real quickly. I know this is sort of off topic here, but I think it's very much relevant. Sort of one of the unintended consequences of this process, is that when particular Medicare plans lose rebates due to the price negotiation, they can put these drugs on a non-tier formulary and basically, loss of access that way is something that I hope that we will all take a hard look at. Make sure that you develop a workaround for that and direct plans to make sure that these drugs, once negotiated, remain available to patients regardless of the plan that they're in.

Similarly, I know that a lot of the community pharmacists are concerned about loss of revenue due to delays in reimbursement for these particular medications once they're negotiated. And a recent survey by the community pharmacists say they may not stock many of these drugs, and given the relatively small population where these medications may be appropriate, I think that's a real concern. So, hope you'll just take a hard look at that as you go through this process. Thanks.

00:38:03

Moderator, RTI International

Thanks for those comments, **[Participant 1]**. I do wanna to go back to one of the things I said in my opening remarks, and that is that for things kind of beyond Ofev and the patient experience with these different conditions that we're talking about today, there is that mailbox available to send those comments to. So, if you do have some policy-related comments and input, that's a good avenue to use for that for sure. Okay, so we already kind of started to get at one of my upcoming questions about main drawbacks or challenges that patients face with Ofev when using it for these conditions. I think we've already heard a decent amount about the drawbacks and challenges the patients face using Ofev for IPF, and a little bit about PPF. Are there other drawbacks or challenges that we haven't already talked about that we should know about for patients using Ofev?

00:39:17

Participant 2 (registered as other)

I don't think so. I think that the biggest thing is just the side effects profile.

00:39:24

Moderator, RTI International

Okay. And I know we didn't talk yet about the big challenges or drawbacks for SSc-ILD. Are there any additional challenges or drawbacks related to using Ofev for that, **[Participant 2]**?

00:39:40

Participant 2 (registered as other)

I think the biggest thing is just that scleroderma patients might have a little bit more gastrointestinal discomfort. And so, because they do have more gastrointestinal issues, the GI side effects are probably a bit worse. I think the other consideration for scleroderma patients, I mean, so Esbriet is

not approved for scleroderma, but if you wanted to call it a therapeutic alternative, even though it's not FDA-approved for scleroderma ILD, you probably wouldn't use [for] scleroderma because of the phototoxicity.

The scleroderma can affect the skin thickness, and so patients sort of are much more sensitive on their skin. And so, you probably wouldn't use it for the phototoxicity period, anyway. So, there's really no therapeutic alternative there.

00:40:27

Moderator, RTI International

Got it. Okay, thank you, **[Participant 2]**. And now let's talk about benefits. I know we've already gotten into this a little bit. But what are the main benefits that patients experience when taking Ofev for IPF—the big benefits of taking Ofev for IPF?

00:40:46

Participant 1 (registered as a representative of a patient advocacy organization)

I would say hope, and hope that they would be able to arrest the progress of their decline. And maybe they're a candidate for transplant, that they would be able to survive. **[Participant 2]** has well-documented, there's nothing pleasant about it. They're not getting better, but they're at least buying some time and hopefully quality time.

00:41:32

Moderator, RTI International

[Participant 2], the main benefits that patients experience when taking Ofev for IPF?

00:41:37

Participant 2 (registered as other)

Yeah, it's slowing the progression of the disease. I think the absolute value is over 100 ccs per year in the 52-week trial.

But I think, in addition to that, prevention of exacerbations. On a population basis, 100 ccs per year doesn't sound like a whole lot on your forced vital capacity, but on an individual level, it is especially because you consider that some of those people that were on placebo probably didn't progress. And the progression was significant in a lot of people. It's the difference between not being on oxygen for your exercise and being on oxygen for your exercise. It's the difference between being on a portable oxygen concentrator and having to use tanks, which prevent you from leaving the house. It's the difference between being able to go to work and having to work from home. It's the difference between being able to take your kids to school and not being able to take your kids to school. It's the difference between thinking you're going to be around for your next birthday or not. It's the difference between needing a transplant earlier and not needing a transplant. That's the difference here.

00:42:55

Moderator, RTI International

Thank you, **[Participant 2]**. And as usual, I'm going to also ask about PPF and SSc-ILD. Are there any different benefits that you would like to call out for those?

00:43:11

Participant 2 (registered as other)

Yeah, it's similar.

00:43:13

Moderator, RTI International

Similar? Okay. I know you all have both mentioned some therapeutic alternatives that can be taken for these conditions in addition to Ofev. Considering patients' experiences with therapeutic alternatives or other medications like Ofev taken to treat IPF, let's start with IPF as usual, how do the benefits of the therapeutic alternatives differ from Ofev, if at all?

So, how are the benefits different between Ofev and its alternatives?

00:43:52

Participant 2 (registered as other)

Yeah, that was basically the same for pirfenidone.

00:43:56

Moderator, RTI International

Okay. Anything else to mention there, **[Participant 1]**?

00:44:00

Participant 1 (registered as a representative of a patient advocacy organization)

No, I don't think so.

00:44:02

Moderator, RTI International

Okay, and what about for the other conditions, PPF, SSc-ILD, any differences in benefits?

00:44:12

Participant 2 (registered as other)

I mean, there's no therapeutic alternatives there. We don't have FDA approval for Esbriet for those conditions. So, I would say the therapeutic, I mean, I guess the therapeutic alternative is, do nothing. So, yeah, it fares poorly, I think is the way to think about it. I mean, if you somehow have pirfenidone but not Ofev on a drug negotiation, then what happens? So okay, you're selecting IPF as the only form of pulmonary fibrosis that you care about, which is problematic, because a lot of people that have pulmonary fibrosis don't have IPF. A lot of people that have pulmonary fibrosis have other forms of pulmonary fibrosis, and so those patients would lose out because there is no antifibrotic for non-IPF except for Ofev.

00:45:11

Moderator, RTI International

Got it, got it, and thanks for the reminder on that, and what about drawbacks? How do the drawbacks or challenges differ between Ofev and therapeutic alternatives for IPF?

00:45:29

Participant 2 (registered as other)

There's a little bit higher rate of cardiac toxicity. So, you just might need to be a little bit more careful with those patients. For whatever reason, some patients might tolerate one more than the other in terms of their gastrointestinal side effects. But, for the most part, I would say that they're fairly similar in terms of side effect profile, and so I don't think that there's a big difference here.

00:46:00

Moderator, RTI International

If you're aware of any patients that have tried multiple medications for treating IPF, what were the reasons for changing medications that you heard about, or that you were involved in? What are some reasons for changing medications for IPF?

00:46:19

Participant 2 (registered as other)

I've seen phototoxicity with pirfenidone, people go on to Ofev. I've seen GI side effects, and then they come down on the dose primarily, and most people can get away with that. I've seen a couple of people switch over earlier on. We don't do that quite as much. We did that sort of earlier on, because both these medicines sort of came to market right at the same time. For the most part, now we will reduce the dose, and then they do fine with the reduction in the dose. We're a little bit more careful about counseling people about the GI side effects before they start. I think that that's just to come with a little bit more knowledge. Sometimes it's patient access. The insurer provides one versus the other. They prefer us to try one versus the other, or something has changed. We need to try the other one. But for the most part, it's not anything dramatic.

00:47:22

Moderator, RTI International

Thanks, **[Participant 2]**. Anything to add to that, **[Participant 1]**, about reasons for changing medications, for treating IPF?

00:47:30

Participant 1 (registered as a representative of a patient advocacy organization)

No, thanks.

00:47:32

Moderator, RTI International

Okay, all right. Well, let's move on. You all have provided a lot of really helpful input so far. So, I really appreciate it. Thank you for that. Now let's switch gears a little bit and talk about how well Ofev and other medications for IPF meet patients' needs.

What would it be like for someone with IPF if Ofev or the other medication that we've discussed were not available? In other words, what needs of people with IPF does Ofev or other medications for this condition meet?

00:48:19

Participant 2 (registered as other)

I think **[Participant 1]** said it earlier, hope. You have a diagnosis. **[MODERATOR]**, you have this irreversible lung disease. It's going to get worse. Guess what? I can do nothing for it.

That was something we were saying. You have this disease, and here's oxygen. You have this disease, and we're going to refer you to transplant. You have this disease, and I can't do anything to stop it or slow it or make it not go away. You have this disease, and you're going to get worse. That's what it was before. And now we at least have something that says you have this disease, and here's something that's going to help prevent it from getting worse. I will say that, having started my career before Ofev, seeing patients come to transplant, we see a lot more patients before that would have these acute exacerbations of their IPF or their progressive pulmonary fibrosis, where they would be stable, and then progress very rapidly. And I don't see it, we still see it once in a while, but the rate of us seeing it, I believe, is much, much lower anecdotally. Because all these patients are now on Ofev. So, I think that that's a big thing. The concern that patients would just be fine and then fall off a cliff is a little bit less of a concern because of these medicines. Until you see somebody who a week ago was hiking a mountain in Hawaii and then is landing in my center on 50 liters of oxygen, and we're evaluating them for transplant. Because of a horrible exacerbation. That potentially could have been prevented. I think that's the difference there.

00:50:26

Moderator, RTI International

Thank you, **[Participant 2]**, and before I turn to **[Participant 1]** to see if he has anything to add, anything additional for the systemic sclerosis-associated?

00:50:36

Participant 2 (registered as other)

Yeah, I think we see less of these major exacerbations with scleroderma or with PPF. We can see them but see less of these major exacerbations. But still the same thing. You have a disease. We can't treat it. That is a hard thing for patients to hear.

00:50:58

Moderator, RTI International

Got it. **[Participant 1]**, anything to add to that?

00:51:00

Participant 1 (registered as a representative of a patient advocacy organization)

No, I think **[Participant 2]** put it pretty well. You would really go back to the world that existed before these medications came on market, and so the hope that I mentioned earlier would be gone. The folks are just going to have to manage as best they can until either they get a transplant or the inevitable. So, it's again, going back to access. It's just critical that the patient and doctor decide they need to be on this medication, it's a wonderful thing that it exists. I wish there were something better and something more hopeful for folks. But this is what we have.

00:51:49

Moderator, RTI International

Your last comment is a great segue into my next question, which is, what aspects of IPF, if any, are Ofev or other medications for this condition, unable to address? What's not being addressed by Ofev and its alternatives?

00:52:09

Participant 2 (registered as other)

Reversal of fibrosis is not being addressed. We need more to slow the progression of fibrosis. Those two things we need. We need better treatments for when patients develop secondary complications. And I think we need better access to oxygen. The SOAR Act was just introduced in the Congress with bipartisan support. We need that to go through. We need that to go through now. We need Congress to act on this. Because our patients are imprisoned in their home.

We need better access to donor organs. We need more donor organs available, so that people can have the life-saving transplant. We need so many things for this condition.

00:53:13

Moderator, RTI International

Would you have anything to add for the progressive pulmonary fibrosis or SSc-ILD for that?

00:53:23

Participant 2 (registered as other)

Progressive pulmonary fibrosis, I think we need similar. All this stuff applies to both of them. With PFF, it's more of a catch-all. So, it can be from any form of interstitial lung disease. So, I don't think there's anything specific to them. With the scleroderma patients, certainly we need better targeted therapies to prevent this from happening in the first place.

We need NIH research dollars. How about that? We need NIH research dollars to help find new drugs to help prevent progression of fibrosis, to develop the development of fibrosis. That's what we need. We need money going there. We need money going to our institutions so that we can actually do the research to prevent this from happening in the first place.

People have a diagnosis, scleroderma. That diagnosis, scleroderma, is associated with the risk of pulmonary fibrosis. We need something that we can give them at that moment, we need to know what's driving it, so we can give them something at that moment, so that their diagnosis of scleroderma doesn't lead to the feared complication of pulmonary fibrosis, doesn't lead to the feared complication of pulmonary hypertension, doesn't lead to them needing a transplant 10, 15, 20 years down the line. That's what we need. And the only way we get that, funding for the NIH, funding for research grants, funding for academic institutions to do what they need to do. And that's what we need.

00:54:57

Moderator, RTI International

Thanks, **[Participant 2]**. What about you, **[Participant 1]**? Aspects of IPF that are not being covered by existing meds?

00:55:07

Participant 1 (registered as a representative of a patient advocacy organization)

Well, I strongly agree with all of that. It is the human impact that you've been told of this illness on, or these illnesses on not just individuals, but their families. So, in addition to everything else that **[Participant 2]** just rattled off, which I totally agree with, is a lot more support for family caregivers and for in-home services and those kind of supports that keep people feeling comfortable and productive as long as possible as they're struggling with a steady decline.

00:55:47

Moderator, RTI International

Thanks, **[Participant 1]**. And oh, sorry, **[Participant 2]**?

00:55:51

Participant 2 (registered as other)

No, no.

00:55:52

Moderator, RTI International

Okay, got it. Okay, thanks again, for all of your input so far. We're getting close to the end of today's session. And I want to wrap things up by talking about your perceptions of the overall importance of Ofev to patients. Thinking about all the different topics that we've already discussed today, how would you summarize the importance of Ofev for people with IPF?

I'll turn to you first this time, **[Participant 1]**.

00:56:23

Participant 1 (registered as a representative of a patient advocacy organization)

It's just critical. There really aren't a whole lot of alternatives out there for folks, and but for the advent of these medications, it was basically a death sentence unless you were able to get a transplant ultimately. It's not much more than where it was before now. It's just the precious time that you can buy with access to these medications. For whatever that's worth. But, I think that's pretty much it for me.

00:57:08

Moderator, RTI International

Thanks, **[Participant 1]**.

How about you, **[Participant 2]**? Thinking about everything we've talked about, how would you summarize the importance of Ofev for people with IPF?

00:57:16

Participant 2 (registered as other)

I mean, I go back to what **[Participant 1]** said earlier. Hope. Until you sit across from a patient and tell them they have a terminal diagnosis, and you can do nothing for it. That's a hard feeling. That's a really hard feeling. And so, we go into medicine because we want to make people better. We go into medicine because we want to impact somebody's life in a positive way. Without this, people that

have pulmonary fibrosis, I don't have things for them. So, it's really hard to say to a patient, I have nothing for you. We're going to keep monitoring you. We'll watch you get worse slowly over time. But I have nothing for you. That's a very uncomfortable place to be on the provider side, and then even more uncomfortable place to be on the patient side. Can you imagine going to your doctor and your doctor tells you, you have a horrible disease that's going to kill you. Worse than cancer, but I got nothing for you. The death from a pulmonary fibrosis is similar to death from lung cancer. Rates of death, pulmonary fibrosis, similar rates of death from lung cancer. But I have nothing for you. Imagine that. Imagine you go to your lung cancer doctor, and you have a new diagnosis of lung cancer. And your doctor says to you, guess what? I have nothing I can give you. That's what it would be.

00:59:04

Moderator, RTI International

Thanks, **[Participant 2]**. Anything to add about the overall importance of Ofev for those other conditions?

00:59:11

Participant 2 (registered as other)

Yeah, I mean, it's the same thing. It's the same thing, actually, probably worse for scleroderma. Rates of progression in scleroderma are really high, because the concomitant other diseases they develop. All these diseases can develop pulmonary hypertension as well, causing heart failure. Without this, we're going to get progressive fibrosis. You're going to get more rates of heart failure, more hospitalizations, more needs for emergency room visits. I mean, I think that that's the other thing here is that, what is the cost to the whole system. If these medicines are not available, the cost of the whole system is that these people are going to progress faster. And then they're going to go to the hospital more. And then they're going to be admitted more in our hospitals, which are already bursting at the seams, and we don't have enough space for the patients that are coming by. We don't have enough beds. Those hospitals that are already bursting at the seams are going to be even more full with patients that have no destination, because when the pulmonary fibrosis gets bad to the point that they're on oxygen in the hospital, there is no destination. We cannot get them home, because they have more oxygen than we can safely provide at home. Or we can get them home, but they cannot leave their bed. They cannot leave their living room. They cannot go out for a walk down the street. They cannot go to work. That's problematic for our society.

01:00:42

Moderator, RTI International

Thanks again, **[Participant 2]**. Okay, well, those are all the main questions I have for you all today. So, anything that we didn't cover in this discussion that you feel is important to share with CMS at this point?

01:00:56

Participant 1 (registered as a representative of a patient advocacy organization)

I don't think so. Thank you. Really appreciate the opportunity.

01:01:03

Moderator, RTI International

Great thanks. Thanks, **[Participant 2]**. Okay, well, thank you again for participating in today's group. We really appreciate you taking the time to talk with me today. Your experiences and input were extremely valuable and will definitely help inform CMS' negotiations for these drugs. CMS staff have been listening to the roundtable and will be able to bring your perspective back to their teams.

[CMS STAFF]?

01:01:32

CMS Staff

Yes, I want to thank you both so much for sharing your experiences with us. We've got a lot that we're going to think about. And I'm just really grateful for your knowledge and time today.

01:01:44

Participant 2 (registered as other)

Sure.

01:01:46

Participant 1 (registered as a representative of a patient advocacy organization)

Thank you.

01:01:48

Moderator, RTI International

Thanks.

Okay, so I just want to point out that address again on this slide. If you have any questions following today's session or wanted to send those types of comments that we talked about, you can submit them to this mailbox here, IRARebateAndNegotiation@cms.hhs.gov, with the subject line 'public engagement events.' Thanks again so much for all of your insights today. I really, really appreciate it, and I hope you all have a good one.

01:02:19

Participant 1 (registered as a representative of a patient advocacy organization)

You, too. Thanks.

01:02:20

Moderator, RTI International

Take care.

===== END OF TRANSCRIPT =====

For a list of the drugs selected for the second cycle of the Medicare Drug Price Negotiation Program, click on the following link. https://www.cms.gov/files/document/factsheet-medicare-negotiation-selected-drug-list-ipay-2027.pdf

For more information on the Medicare Drug Price Negotiation Program, please click on the following link. https://www.cms.gov/priorities/medicare-prescription-drug-affordability/overview/medicare-drug-price-negotiation-program

Appendix

Participant 1: Registered as a representative of a patient advocacy organization

Declared Conflicts of Interest	
Yes	Receipt of financial payments (e.g., gifts, funding, research support, honoraria, travel, or other expenses) from a company with direct/indirect interest in the Negotiation Program, in excess of $10,000 by you, your spouse, or an immediate family member
No	Direct assistance preparing your remarks from someone who is NOT a family member, caregiver, friend, or your healthcare provider
No	You, your spouse, or an immediate family member is employed by or holds equity interest (stock or ownership interest) in excess of $10,000 in a company or related association with direct or indirect interest in the Negotiation Program
No	Any other personal or professional relationship or interaction with a company or related association with direct or indirect interest in the Negotiation Program that may be considered a financial conflict of interest

Participant 2: Registered as other

Declared Conflicts of Interest	
N/A*	Receipt of financial payments (e.g., gifts, funding, research support, honoraria, travel, or other expenses) from a company with direct/indirect interest in the Negotiation Program, in excess of $10,000 by you, your spouse, or an immediate family member
N/A*	Direct assistance preparing your remarks from someone who is NOT a family member, caregiver, friend, or your healthcare provider
N/A*	You, your spouse, or an immediate family member is employed by or holds equity interest (stock or ownership interest) in excess of $10,000 in a company or related association with direct or indirect interest in the Negotiation Program
N/A*	Any other personal or professional relationship or interaction with a company or related association with direct or indirect interest in the Negotiation Program that may be considered a financial conflict of interest

**Participant did not select either "Yes" or "No" to a conflict of interest.*